

Safe, efficient creation of human induced pluripotent stem cells without the use of retroviruses

## Grant Award Details

Safe, efficient creation of human induced pluripotent stem cells without the use of retroviruses

Grant Type: New Cell Lines

Grant Number: RL1-00634

Investigator:

Name:	Michele Calos
Institution:	Stanford University
Type:	PI

Disease Focus: Immune Disease

Human Stem Cell Use: iPS Cell

Cell Line Generation: iPS Cell

Award Value: \$1,406,875

Status: Closed

## Progress Reports

Reporting Period: Year 1

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Reporting Period: Year 3

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## Grant Application Details

Application Title: Safe, efficient creation of human induced pluripotent stem cells without the use of retroviruses

**Public Abstract:**

Embryonic stem cells open up exciting new prospects for medicine, because they can differentiate into any tissue in the body. Therefore, they have the potential to be used to repair faulty tissues in diseases like diabetes, heart disease, and neural disorders. Furthermore, stem cells can be corrected by gene therapy and transplanted, in order to treat a wide variety of genetic diseases, such as sickle cell anemia. However, embryonic stem cell research has been difficult because of the technical and ethical problems involved in obtaining these cells from human embryos, as well as the need to transplant cells that are immune-matched to the recipient patient. The solution to these challenging biological and ethical problems may emerge from recent findings that show that cells that behave like embryonic stem cells can be derived from ordinary cells easily obtained from a patient, such as skin cells. This process is known as reprogramming. This breakthrough means that embryos may no longer be required to generate the stem cells needed for exciting new therapies. However, the methodology that is currently used to create reprogrammed cells involves introducing many viruses into patient cells. This procedure is itself dangerous and can lead to tumors and other abnormalities in the cells. As a result, the reprogrammed cells created to date are not suitable for use in the clinic. This proposal seeks to solve this problem by creating a novel method for introducing the reprogramming genes into one safe place in the chromosomes that will have no adverse effects on the cells. In these experiments, a simple, safe way to make reprogrammed cells without viruses will be developed. The reprogrammed cells made by this method will be thoroughly tested to ensure that they have all the beneficial properties of embryonic stem cells and are safe to use. The emphasis will be on generation of human reprogrammed cells that are safe and effective in therapies. Reprogrammed mouse cells will also be generated, for use in testing in mice before human clinical trials. The reprogrammed cells will be evaluated for their ability to differentiate in culture into tissues such as nerve, heart, and blood cells. The cells will then be tested for their capacity to cure a genetic disorder in a mouse model. Success in these experiments will provide a simple and safe method to generate reprogrammed stem cells and will speed the use of these cells in a wide variety of clinical applications.

**Statement of Benefit to California:**

Human pluripotent stem cells derived from ordinary adult cells are a scientific breakthrough that could speed medical advances to the public. These "reprogrammed" stem cells, made from ordinary cells, could remove the technical and ethical impasses that have delayed advances with stem cells that are derived from human embryos. However, current methods to make reprogrammed cells utilize viruses that are themselves dangerous. This proposal will apply new, California-invented technology, in the form of a novel gene addition system, to create an easy and safe method to make reprogrammed stem cells without the use of viruses. By replacing the current ~20 random viral integration sites with one safe, defined integration site, the resulting stem cells are likely to be suitable for clinical use, without the fear of tumors or other abnormalities. This project is feasible in all its elements, highly relevant to the goals of CIRM, and will result in a variety of new lines of pluripotent human stem cells. The availability of high-quality stem cells, made from ordinary patient tissue, will allow researchers to move more quickly to develop safe, effective, stem cell therapies for the people of California. This highly innovative project could create a leap forward for the entire stem cell field and greatly speed clinical applications. Therefore, this application is of great importance to California.

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